

Single Day Event

**Emerging Therapeutic Technologies in
Inflammation and Immunology (I&I)**

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Agenda

Introduction to Inflammation and Immunology (I&I) Therapeutic Area

Diseases under the I&I

Endpoints

CRO/PRO Domains

Current and Emerging Data Analysis

Traditional Treatments

Current Treatments

Emerging and Future Technologies

Impact of Emerging Technologies on Clinical Data Analysis

Conclusion



Inflammation and Immunology (I&I)



Inflammation and Immunology (I&I) refers to the study and treatment of diseases where the immune system (Abnormal Immune response) and inflammatory responses (Chronic Inflammation) are involved.



Focus on understanding how the immune system and inflammation contribute to diseases like autoimmune disorders, inflammatory diseases, and immune-related cancers.



Why Emerging Technologies Matter:

Need for innovative treatments to address complex, chronic diseases

Emerging technologies offer precision, better efficacy, and fewer side effects.

Autoimmune Diseases

Rheumatoid Arthritis

Early Intermediate Late



Rheumatoid Arthritis (RA): Affects the joints, causing pain, swelling, and stiffness



Lupus (Systemic Lupus Erythematosus): Can affect the skin, joints, kidneys, brain, and other organs



Psoriasis: Causes rapid skin cell production, leading to scaly patches on the skin



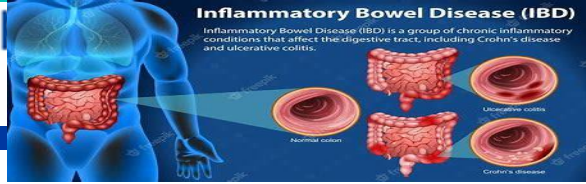
Vitiligo: Affects the skin, causing the loss of pigment in certain areas, leading to lighter patches of skin.

Multiple Sclerosis (MS)



Multiple Sclerosis (MS): Targets the protective covering of nerves, leading to neurological symptoms

Inflammatory Diseases



Inflammatory Bowel Disease (IBD): Includes Crohn's disease and ulcerative colitis, affecting the digestive tract



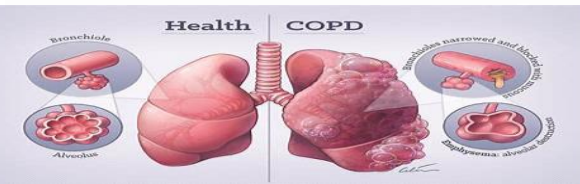
Atopic Dermatitis (AD): It is a chronic (long-term) condition that causes inflammation, redness, itching, and dryness of the skin



Gout: A type of arthritis caused by the accumulation of uric acid crystals in the joints



Ankylosing Spondylitis: Affects the spine and large joints, causing pain and stiffness



Chronic Obstructive Pulmonary Disease (COPD): Involves chronic inflammation of the airways, often due to smoking



Clinical Endpoints

- **Disease Activity Scores:** DAS28 (RA), PASI (Psoriasis), SLEDAI (Lupus), CDAI (IBD), VASI (Vtiligo)
- **Quality of Life Measures:** SF-36, EQ-5D, WPAI (Work Productivity and Activity Impairment)
- **Physical Function Tests:** 6-minute walk test, Joint Flexibility Tests
- **Imaging-based Assessment:** MRI for joint damage, Endoscopy for IBD
- **Biomarkers (Exploratory analysis):** Inflammatory markers like CRP (C-reactive protein), TNF-alpha levels, RF (Rheumatoid Factor) and interleukins.
- **Pharmacokinetics & Pharmacodynamics:** Drug absorption, metabolism, and immune modulation
- **Safety Endpoints:** Adverse Events, Laboratory Tests, etc



Clinician reported outcome (CRO) and Patient reported outcome (PRO)

ClinRO	RA: Disease Activity Score (DAS28), Simplified/Clinical Disease Activity Index (CDAI/SCDAI) Systemic Lupus Erythematosus (SLE): SLE Disease Activity Index (SLEDAI), Physician Global Assessment (PGA) Crohn's Disease & Ulcerative Colitis (IBD): Crohn's Disease Activity Index (CDAI); Ulcerative Colitis Disease Activity Index(UCDAI)
Quality of Life (QoL)	General QoL: Tools like the Short Form Health Survey (SF-36) or the EuroQoL-5D (EQ-5D) assess overall health and well-being Disease-Specific QoL: Instruments like the Inflammatory Bowel Disease Questionnaire (IBDQ) for IBD or the Rheumatoid Arthritis Quality of Life (RAQoL) questionnaire
Symptom Severity	Pain: Visual Analog Scale (VAS) or Numeric Rating Scale (NRS) for pain intensity. Fatigue: Fatigue Severity Scale (FSS) or the Multidimensional Assessment of Fatigue (MAF) scale
Functional Status	Physical Function: Health Assessment Questionnaire (HAQ) for rheumatoid arthritis or the Crohn's Disease Activity Index (CDAI) for Crohn's disease Daily Activities: Assessments of the impact on daily living activities, such as the Bath Ankylosing Spondylitis Functional Index (BASFI) for ankylosing spondylitis
Emotional and Mental Health	Anxiety and Depression: Hospital Anxiety and Depression Scale (HADS) or the Patient Health Questionnaire (PHQ-9) for depression Emotional Well-being: Tools like the Psychological General Well-Being Index (PGWB)
Treatment Satisfaction	Satisfaction with Medication: Surveys assessing satisfaction with treatment effectiveness, side effects, and overall experience (TSQ) Adherence to Treatment: Questionnaires evaluating how well patients follow their prescribed treatment regimens (MMAS)



Analysis Methods

Existing Analytical Methods

Descriptive & Inferential Statistics: Mean, Median, T-tests, ANOVA

Regression Models: Logistic and Linear Regression for disease progression

Survival Analysis: Kaplan-Meier curves for treatment effectiveness

Estimands: The Estimand framework provides a systematic approach to the definition of the treatment effect under investigation in a clinical trial.

Emerging Analytical Methods

Artificial Intelligence & Deep Learning: Pattern recognition in imaging & biomarker prediction, Generative AI

Wearable Data Analytics: Continuous tracking of disease activity using smart devices. Provides real-time data on patient health and activity

Real-World Evidence (RWE) & Big Data Analytics: Utilizing EHRs and patient registries for long-term disease monitoring



Traditional Treatment



Conventional Treatments:

Non-Steroidal Anti-Inflammatory Drugs (NSAIDs):

For pain and inflammation relief (Ibuprofen, Aspirin etc).

Corticosteroids: Reducing inflammation and immune suppression (Prednisone, Hydrocortisone etc).

Immunosuppressants: Methotrexate, Azathioprine.



Limitations:

Side effects (e.g., infections, organ damage).

Long-term treatment failure or resistance.

Generic treatment pathways not addressing underlying immune mechanisms.



Current Technologies

Biologic Therapies

Monoclonal Antibodies: These target specific components of the immune system. Examples include TNF inhibitors (e.g., infliximab, adalimumab) for rheumatoid arthritis and IBD

Interleukin Inhibitors: These block specific interleukins involved in inflammation, such as IL-6 inhibitors (e.g., tocilizumab) and IL-17 inhibitors (e.g., secukinumab)

Small Molecule Drugs

Small molecule drugs are more traditional oral therapies, often targeting intracellular signaling pathways or enzymes that modulate immune function.

JAK Inhibitors: These inhibit Janus kinase enzymes, which play a role in the inflammatory process. Examples include **Tofacitinib, Baricitinib and Upadacitinib**

Phosphodiesterase-4 (PDE4) Inhibitors: cAMP pathway in immune cells to reduce inflammation. Example **Apremilast** for psoriasis and psoriatic arthritis

Novel Drug Delivery Systems

Nanotechnology: Utilizing nanoparticles to deliver drugs directly to inflamed tissues, enhancing efficacy and reducing side effects

Biodegradable Implants: Implants that release anti-inflammatory drugs over time at the site of inflammation

Lifestyle and Complementary Therapies

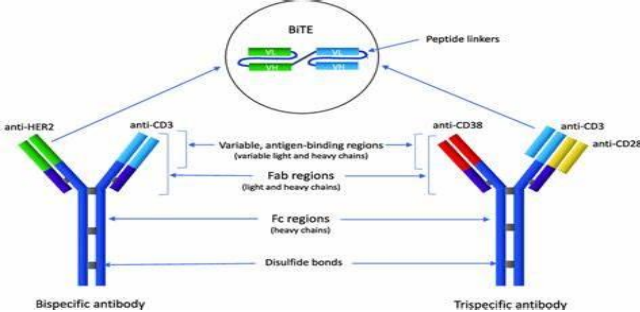
Diet and Nutrition: Anti-inflammatory diets and supplements (e.g., omega-3 fatty acids) to manage symptoms

Physical Therapy and Exercise: Tailored exercise programs to improve mobility and reduce inflammation



Gene Editing and Gene Therapy

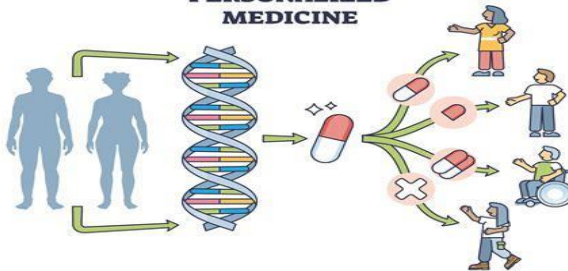
- **CRISPR-Cas9:** Precise gene editing for correcting genetic defects.
- **Gene therapy:** Delivering therapeutic genes to modulate the immune response.



Advanced Biologics

- **Bispecific and Trispecific antibodies:** Targeting two/Three antigens simultaneously.
- **Nanobodies:** Smaller antibody fragments with better tissue penetration.

PERSONALIZED MEDICINE



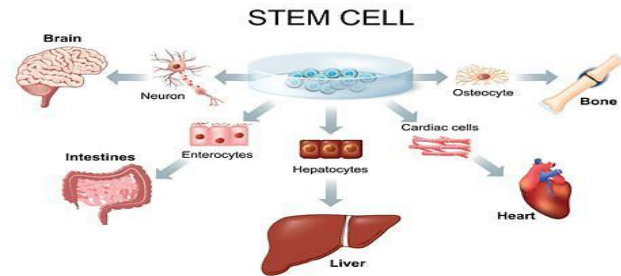
Precision/Personalized Medicine

- **Genomic and Proteomic Profiling:** Using OMIC data to tailor treatments based on individual genetic and protein expression profiles
- **Targeted Therapies:** Repurposing precision oncology technologies to develop targeted treatments for I&I diseases



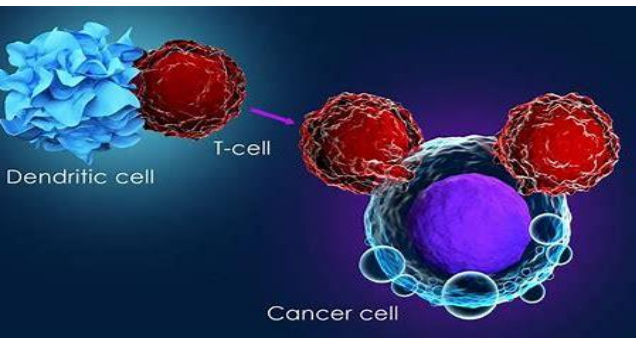
Artificial Intelligence (AI) and Machine Learning

- **Predictive analytics:** Predicting disease flares and personalizing treatment.
- **Drug discovery:** Identifying new drug candidates.



Regenerative Medicine

- **Tissue engineering:** Creating bioengineered tissues and organs.
- **Advanced stem cell therapy:** Repairing and regenerating damaged tissues



Immuno-oncology

- Leveraging immunotherapy in cancer treatment, with monoclonal antibodies and CAR-T cells.



Challenges and Impacts

Regulatory and Ethical Considerations and Challenges

- **Regulatory Hurdles:** Approval processes for gene therapies, biologics, and AI-based diagnostics
- **Clinical Trials and Safety Concerns:** Risk of adverse effects and the long process of proving efficacy
- **Cost and Accessibility:** High cost of advanced therapies like biologics, gene editing, and AI-driven treatments
- **Data Privacy:** Handling sensitive genomic and health data while adhering to regulatory standards (e.g., HIPAA, GDPR).
- **Bias in AI Models:** AI models can inadvertently perpetuate biases if not trained on diverse and representative datasets.

Impact of Emerging Technologies on Clinical Data Analysis

- **Data Complexity**
 - Emerging therapies, especially biologics and gene therapies, generate vast and complex datasets (e.g., genomic, proteomic, patient health data, clinical trial data). This is challenging but crucial for identifying treatment efficacy and safety
- **Real-Time Data**
 - Technologies like wearables and remote patient monitoring provide **real-time** health data.
 - Continuous patient data must be integrated and analyzed to adjust treatment protocols dynamically
- **Personalized Medicine**
 - Genetic and Biomarker Data are increasingly used to customize treatments.
 - Clinicians rely on advanced algorithms and AI to analyze diverse datasets and determine the best course of action for individual patients.
- **Predictive Modeling**
 - Machine learning algorithms help predict outcomes, side effects, and the likelihood of treatment success.



Summary

Key Takeaways:

- Emerging therapeutic technologies are transforming the landscape of treatments, offering more targeted, personalized, and efficient treatment options.
- These technologies generate complex data that requires advanced analytics to optimize treatment outcomes and clinical trial designs.
- Future developments will rely heavily on innovations in data science, AI, and real-time monitoring to enable better patient care and more efficient clinical research.
- Collaboration between researchers, clinicians, and technologists is essential for the next breakthroughs in treatment.

Final Thought: The future holds promise for more effective, personalized, and less invasive therapies across all areas of healthcare.



Reference

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- [Applications of emerging extracellular vesicles technologies in the treatment of inflammatory diseases - PMC](#)
- [Recent advances in immunotherapies against infectious diseases – PMC](#)
- Google for various images and generic information



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Thank you



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